
Three embryonic stem cell trials and counting

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We're back after a vacation filled with news about the second ACT embryonic stem cell trial getting FDA approval earlier this week. This one is for macular degeneration. Their first trial, approved by the FDA on November 22, was for Stargardt's macular degeneration. That brings the total to three trials testing therapies based on embryonic stem cells.

For anyone who missed the ACT announcement in the midst of New Year's excitement, here's a good story from the MIT Technology Review.

It's easy to get excited about this progress, and about the additional embryonic and adult stem cell trials I hope to see approved and started in the next year. Whatever the stem cell type, progress toward new therapies is something to celebrate. At the same time I do worry about the level of hope being placed on these first three trials. More early stage trials fail than succeed, and it is likely that at least one of these early embryonic stem cell trials will fail too. That's the whole point of testing therapies in humans - no matter how effective a therapy might have been in mice or other animals, we humans respond differently and you just never know what to expect.

The Technology Review story had this to say about the many unknowns of the ACT trial:

“ A number of questions remain to be answered, including how well the cells will survive in a diseased eye. Increasing evidence suggests that macular degeneration is in part an immune defect, and some people with the disease have signs of inflammation in the retina, which may make it more difficult for the implanted cells to take root. "That's something animal models haven't been able to look at carefully," says Reh.

It's also not yet clear whether, if the cells do survive, they will delay or prevent further vision loss, or actually improve vision. Transplants of retinal pigment epithelium cannot replace lost photoreceptors, but they may help damaged photoreceptors function better and in turn enhance vision.

I should add that the story had a lot to say about why the trial might succeed, too.

I'm optimistic that some of these early stem cell trials will go on to be successful, and that some of the work CIRM has already funded will be making its way to trials shortly. I also hope people remember that when some of these trials aren't successful the first time it doesn't invalidate stem cell therapies. It just means stem cells are as difficult to bring to the clinic as vaccines or cancer chemotherapeutics or any of the many other therapies that now save lives every day.

- A.A.

Tags: FDA, macular degeneration, clinical trials, spinal cord injury

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